October 18, 1999

Contact: HRSA Press Office
(301) 443-3376

IMPROVING THE NATION'S ORGAN TRANSPLANTATION SYSTEM

Background

Since the 1970s, when organ transplantation became an established medical procedure, the number of organ transplants performed each year in the United States has grown from 12,618 in 1988 to 20,961 in 1998; and the number of centers performing this surgery has grown from 235 in 1988 to 278 today. The number of patients awaiting transplantation has grown even more rapidly: from about 14,000 in 1988 to some 66,000 persons on waiting lists for organ transplantation today, ranging in condition from non-urgent to extremely urgent. However, organ donation has grown much more slowly -- from 5,906 donors in 1988 to 9,913 in 1998. Almost 5,000 patients die each year, some 13 each day, while awaiting an organ for transplantation.

Problems in the Transplantation System

While progress in transplant medicine represents a remarkable success story, the nationwide network that binds together our transplant system could work better, according to conclusions of a landmark study this year by the National Academy of Science's Institute of Medicine, *Organ Procurement and Transplantation*. In particular, since there are too few organs available to provide for all patients, the Organ Procurement and Transplantation Network (OPTN) needs to allocate organs in as medically effective and fair a manner as possible, to save lives and make best use of this scarce resource. Problem areas include:

- Arbitrary Boundaries Sometimes Prevent Organs from Reaching Most Urgent Patients -- Under current organ allocation policies, patients are given priority for organs based first on their geographic location instead of their medical need. These "local first" rules can mean that a less medically urgent patient receives a transplant, while another more urgent patient who could receive the organ dies. In fact, in 1998, some 71 percent of livers were transplanted to patients in the least urgent medical status categories, while in the same year 1,300 patients died while waiting for a liver. This year's Institute of Medicine report concluded that organ sharing (in particular, livers) must take place over a sufficiently large population area to ensure that those life-saving organs can reach the medically urgent patients who need them most, and for whom transplantation is medically appropriate. While there are time restrictions on how long a donated organ can remain viable for transplantation, these times have lengthened as medical technology has improved. In addition, some organs

have much longer "cold ischemic times" than others. *The OPTN needs to develop allocation policies that provide organs, whenever possible, to the patients who need them most. Allocation policies should be based on medical criteria, including organ viability. Organs available for transplantation should not be restricted by arbitrary "local first" allocation policies that no longer have a medical basis.*

- Inconsistent Medical Criteria Are Unfair to Patients and Fracture the Transplant System -- In order to achieve fair and medically effective use of scarce organs, transplant centers and physicians must have uniform criteria for deciding when to list patients for transplantation and for identifying patients' medical urgency status. In the absence of enforceable uniform criteria, the system is vulnerable to "gaming," and the necessary trust between different centers is threatened. This situation is exacerbated insofar as the transplant network may use a patient's cumulative waiting time as an allocation criterion, since this may create an incentive to put a patient on the waiting list earlier than medically necessary, in order to build up time. *Organ allocation should be based primarily on medical need, and the OPTN needs to develop uniform medical criteria and the means for ensuring compliance with them.*

- IOM Points Out OPTN Data Are Incomplete and Non-current -- Drawing attention to the "paucity" of current transplantation data available from the transplantation network, the Institute of Medicine report said thorough and current transplant data is needed both to inform doctors and patients, and to enable transplant professionals to determine best medical practices in the transplant field. The OPTN has made some progress in this area even since the IOM issued its report. *With on-line data reporting capabilities, the OPTN should be able to provide much more timely and thorough information.*

- Lack of Oversight Contributes to Contention and Confusion -- The Institute of Medicine report called on HHS to exercise the oversight responsibilities assigned to it by the National Organ Transplant Act "to manage the system of organ procurement and transplantation in the public interest." Lack of clarity as to the federal role, to bring about public accountability, has contributed to confusion and distrust within today's organ transplant system. *Responsibility for the national system established under NOTA is shared by HHS and the transplant community. The day-to-day operation of the system is the responsibility of medical professionals. The development of the OPTN's medical and allocation policies is the responsibility of transplant professionals, in particular transplant physicians, in cooperation with the centers, patients and donor families represented on the OPTN board. The HHS responsibility, as recommended by the Institute of Medicine, is to provide for public accountability, including performance goals for the system, as well as review and approval of OPTN policies on the basis of those goals. HHS' role, to assure that the system operates in the public interest, is exercised on behalf of patients who rely on the fairness and effectiveness of OPTN policies; on behalf of donors who provide organs in trust to the system; on behalf of transplant centers, which are required to adhere to OPTN policies; and on behalf of taxpayers, who pay for half of all transplant costs in the United States.*

The primary problem remains the shortage of organs available for transplantation. HHS, the transplant community and dozens of partner organizations are carrying out a National Organ and Tissue Donation Initiative aimed at increasing donation. Donation increased 5.6 percent in

1998, the first significant increase in three years. These efforts are continuing. In addition, HHS supports development of improved anti-rejection medications, as well as other research to help those needing organ transplantation.

OPTN Final Rule -- Legislative History

The National Organ Transplant Act of 1984 was enacted to create a system that would allocate donated organs fairly among patients who needed them, using medical criteria. Before NOTA was enacted, there were few rules to assure that organs were provided to those who needed them most . NOTA was intended to create a system in which consistent medical criteria would be developed and used by transplant professionals, with oversight by the Secretary of Health and Human Services to help ensure that the national system created by the Act would operate in the public interest.

The need for HHS review of transplant network policies was cemented in 1986 with enactment of Section 1138 of the Social Security Act. This measure gave OPTN the authority to require transplant centers to comply with its policies, making compliance a condition for hospital participation in Medicare and Medicaid. Since decisions on Medicare and Medicaid participation are reserved under law for the HHS Secretary, OPTN policies would require Secretarial review and approval before they could be legally binding on transplant hospitals.

In 1994, HHS published a proposed OPTN regulation, beginning the process of formally outlining the joint roles to be played by OPTN and HHS. Public hearings were held, and a new comment period on this proposed regulation was provided in 1996. The final rule, with an additional comment period, was published April 2, 1998. Throughout this development period, HHS aimed at making clear the separation between the federal role in review and approval of major policies, as opposed to the responsibilities of the transplant community to develop the policies and provide appropriate medical care to patients. The HHS role, as noted in the IOM report, is to ensure that the transplant system functions in the public interest, and to avoid interference in the practice of medicine.

In October 1998, Congress postponed implementation of the final rule for a year, making time for an independent study by the Institute of Medicine, development of improved center-specific data, and continued discussions between HHS and various members of the transplant community, including the OPTN contractor, transplant physicians and other health professional and patient representatives. The IOM report was published in July; new center-specific data was provided by HHS to Congress this month; and meetings with representatives of 11 transplant organizations took place throughout the year, including a meeting Sept. 15 with representatives of the organizations meeting as one group.

As a result of the IOM study and the discussions, as well as written comments on the final rule, amendments are being published today, which make improvements to the regulation while at the same time preserving and clarifying the intent and principles.

OPTN Final Rule -- Making Improvements While Preserving Core Features

Amendments published today improve and refine the Final Rule. They make much clearer the separation of roles between the federal government and the transplant professionals who make the medical decisions. They add a significant new element, an independent advisory committee to assure that HHS has the best scientific counsel as it oversees the broad policies of the Organ Procurement and Transplantation Network, consistent with the law. They provide for appropriate representation by transplant physicians and surgeons, and by patient and donor representatives, in the leadership of the OPTN. They envision a more vigorous role for the OPTN in assuring that consistent medical criteria are used, so that we can be assured that organs are being allocated in the fairest and most medically effective manner. And they provide expanded flexibility for development of performance criteria, including modifications in keeping with the IOM's recommendations concerning the use of waiting time as a performance measure. At the same time, the fundamental principles and core provisions of the OPTN final rule remain:

- <u>OPTN to Develop Standardized and Enforceable Medical Criteria</u> -- To achieve fair allocation of organs and best medical use of the organs, physicians and transplant centers need to be using uniform criteria for placing patients on the waiting lists and defining their medical urgency status. Without such uniformity, there cannot be assurance that patients who most urgently need a life-saving transplant will receive one. *Under the final rule, these criteria would be developed by the OPTN (not HHS). Likewise, the final rule calls on the OPTN to develop means for enforcing these criteria, so that OPTN can prevent organs from being misallocated, and can discipline for any individuals or centers who try to "game" the system by describing their patients as more medically urgent than they really are. <u>The need for enforceability is strengthened in today's amendments (see below).</u>*

- <u>OPTN to Develop Allocation Criteria to Provide for Adequately Broad Sharing</u> -- As reinforced by the findings of the IOM report, sharing of organs (in the report, specifically livers) needs to be over a sufficiently large population area to ensure the likelihood that the organs can reach those patients who need them most. The transportation of organs to the best patient candidate should not be impeded by arbitrary, non-medical boundaries. *Under the final rule, these allocation policies would be designed and proposed by the OPTN, taking into account the optimal period for transplantation to patients and the need to preserve the viability of organs, and other medical considerations. <u>The intent of these provisions, including the meaning of medical urgency and the requirement that sound medical judgment be exercised and organs not be wasted in futile transplantations or excessive transportation times, is clarified in today's amendments (see below).</u>*

- <u>OPTN and Medical Decision-Making Distinct from HHS Responsibilities</u> -- As recommended by the IOM report, an important role should be played by HHS in overseeing the transplantation system to help ensure that it is operating in the public interest, and that its policies result in organ allocation and transplantation that are as fair and as medically effective as possible. *Under the final rule, HHS does not interfere with case-by-case medical decision-making. Likewise, broad medical policies are to be designed by the OPTN, to be*

enforceable on transplant centers and physicians once they are approved by the HHS Secretary. Today's amendments make this separation of roles clearer. As recommended by the IOM, they also add a new advisory committee to ensure that the Secretary has the best scientific advice in reviewing OPTN policies (see below).

- OPTN to Design Performance Criteria -- As recommended by the IOM report, HHS should establish goals for the transplantation system, including "patient-centered, outcome-oriented performance measures." *Under the final rule, HHS sets broad goals, especially reduced variability among transplant centers. In general, this means the transplantation system should function to allow organs to reach patients who need them most, with high-quality treatment at all centers -- so that, as much as possible, patients should not be disadvantaged by listing in one center or another. The final rule calls on the OPTN to design and propose the specific performance criteria, for review and approval by the Secretary. Today's amendments expand the OPTN's flexibility in designing these criteria; and in particular, they reduce specific reliance on waiting times, as recommended by the IOM report. Ultimately, no use of waiting times as a performance measure would be required if the OPTN decides to cease using waiting times as a criteria in allocating organs (see below).*

- OPTN to Provide Improved Data -- As reinforced by the findings of the IOM report, the OPTN should provide more current, complete and usefully organized data for patients, physicians and the transplant community, including payors and researchers. A special analysis prepared by HHS in response to the Congressional mandate shows that center-specific information can be provided that will better help the transplant profession to identify best practices and thus improve outcomes for patients. *Under the final rule, specific data requirements and timetables are laid out. The amendments make some minor alterations in the timetables, but preserve the data requirements (see below).*

OPTN Final Rule -- Amendments

Amendments to improve and clarify the final rule include the following:

1. OPTN Enforcement of Standardized Criteria -- In addition to requiring OPTN to develop standardized criteria for listing patients and defining their medical urgency status, it is important to ensure compliance with these standardized criteria when organs are allocated. This is fundamental to fairness in the system. OPTN should have means for ensuring compliance, both retrospectively and prospectively, to prevent organs from being misallocated due to incorrect status descriptions. A new Section 121.8(a)(7) is added to the final rule to encourage OPTN to develop appropriate enforcement means. HHS plans to work with OPTN toward such mechanisms.

2. Allocation Criteria Clarifications -- Considerable confusion was generated concerning provisions in the final rule which were intended to ensure that organs are allocated in the best way medically possible to save lives. The final rule is amended in several areas to make it even clearer that: providing organs wherever possible to most medically urgent patients does not mean transplanting to patients too ill to benefit, but rather only to patients for whom

transplant is medically appropriate; allocating organs to most medically urgent patients does not require transporting organs so far that organ viability would be threatened, but instead recognizes that medical factors limit the transportability of organs; the final rule does not require a single "national list" for allocation, but rather calls on the OPTN to develop adequately broad allocation areas to ensure best use of organs to save lives, as recommended by the IOM report; adequately broad sharing of organs is not expected to negatively impact small or medium sized transplant centers delivering good quality care to patients for whom transplant is medically appropriate, and the final rule specifically provides for monitoring any unexpected impact on these centers so that access to good quality transplant care is not reduced. In addition, language is added to make clear how the final rule provisions apply to kidney matches.

3. Advisory Committee -- The IOM report recommended establishment of an independent scientific review board "for assisting the Secretary in ensuring that the system of organ procurement and transplantation is grounded on the best available medical science and is as effective and as equitable as possible." In response to this recommendation, as well as comments received, HHS intends to create an Advisory Committee on Organ Transplantation, and to implement the IOM's recommendation that this committee have several key responsibilities. In the final rule, Section 121.12 has been revised to provide for establishment of this committee. Revisions are also at Sections 121.4(b)(2) and (d).

4. Federal Responsibilities -- With the addition of the Advisory Committee, the final rule retains its provisions outlining the responsibilities of the HHS Secretary for reviewing and approving major policies designed by the OPTN. These provisions are consistent with the recommendations of the IOM report. The final rule makes clear that it is not the desire nor intention of HHS to interfere in the practice of medicine. The HHS responsibility is to ensure that the broad policies of the OPTN create an equitable and medically effective system, and to review and approve such policies before they become enforceable by the OPTN. One amendment provides that OPTN must provide proposed policies to the Secretary at least 60 days, instead of 30 days, before proposed implementation.

5. Performance Criteria -- Amendments to the final rule provide added flexibility for the OPTN in designing performance criteria. Analyses performed by HHS and provided to Congress and to the OPTN this month point the way toward potential use of new medically-appropriate performance measures. The final rule published April 2 placed specific emphasis on reducing variability among transplant centers in waiting times as a performance indicator. This was in part because waiting times are used by the OPTN as an allocation criterion, and in part because other data have not been available. The IOM report concluded that waiting times are of limited usefulness in allocation of organs, and it recommended that OPTN discontinue use of waiting time as an allocation criterion for less urgent patients. Consistent with this recommendation, the final rule would no longer require use of waiting times as a performance criterion wherever the OPTN did not use waiting time as an allocation criterion (Section 121.8).

6. Data Requirements -- Consistent with the IOM report findings, data requirements in the final rule are left mostly unchanged. The OPTN is given until June 2000 to comply with the new

requirements, and requirement for reporting every six months may be waived as appropriate by the Secretary under the amendments. (Section 121.11(b))

7. Board Composition -- The amendments make alterations in the requirements for composition of the OPTN Board of Directors. In response to comments noting the importance of medical expertise, the Board membership is put at approximately 50 percent physicians and transplant surgeons, instead of "no more than 50 percent," with at least 25 percent representation from patients, recipients, donors and family members. In addition, these approximate representations are required for the OPTN Executive Committee, raising patient/donor representation there. (Section 121.3)

8. Socioeconomic Barriers -- The final rule includes provisions directing OPTN to investigate means for reducing socioeconomic barriers to transplantation. While this remains unchanged, the amendments make clear that HHS is not asking transplant centers to bear the costs of transplantation for poor patients. Transplant centers, however, may be able to waive payment of the initial registration fee to get on a waiting list. (Section 121.4)

In addition, in the preamble to the amendments, HHS invites the OPTN to develop ideas for rewarding high-performing organ procurement organizations. Some OPOs have markedly superior rates of donation and donor consent, and HHS is anxious to provide incentives for high organ donation rates.

In order to provide time to review the amendments, the effective date of the final rule (as amended) is delayed until 30 days after publication in the *Federal Register*.

IOM Report -- Major Recommendations

The Institute of Medicine issued its report, *Organ Procurement and Transplantation*, on July 22. While the conclusions of the report support the thrust and provisions of the OPTN final rule, many of the amendments made today reflect refinements suggested by the IOM report. A significant addition to the regulation, a scientific advisory committee to ensure best counsel for the Secretary, was suggested by the IOM report. The report's major recommendations:

"RECOMMENDATION 1: Establish Organ Allocation Areas for Livers. The committee recommends that the DHHS Final Rule be implemented by the establishment of Organ Allocation Areas (OAAs) for livers - each serving a population base of at least 9 million people (unless such an area would exceed the limits of acceptable cold ischemic time). OAAs should generally be established through sharing arrangements among organ procurement organizations to avoid disrupting effective current procurement activities.

"RECOMMENDATION 2: Discontinue Use of Waiting time as an Allocation Criterion for [Liver Transplant] Patients in Statuses 2B and 3. The heterogeneity and wide range of severity of illness in statuses 2B and 3 make waiting time relatively misleading within these categories. For this reason, waiting time should be discontinued as an allocation criterion for

status 2B and 3 patients. An appropriate medical triage system should be developed to ensure equitable allocation of organs to patients in these categories. Such a system may, for example, be based on a point system arising out of medical characteristics and disease prognoses rather than waiting times.

"RECOMMENDATION 3: Exercise Federal Oversight. The Department of Health and Human Services should exercise the legitimate oversight responsibilities assigned to it by the National Organ Transplant Act, and articulated in the final rule, to manage the system of organ procurement and transplantation in the public interest. This oversight should include greater use of patient-centered, outcome-oriented performance measures for OPOs, transplant centers, and the OPTN.

"RECOMMENDATION 4: Establish Independent Scientific Review. The Department of Health and Human Services should establish an external, independent, multidisciplinary scientific review board responsible for assisting the Secretary in ensuring that the system of organ procurement and transplantation is grounded on the best available medical science and is as effective and as equitable as possible.

"RECOMMENDATION 5: Improve Data Collection and Dissemination. Within the bounds of donor and recipient confidentiality and sound medical judgment, the OPTN contractor should improve its collection of standardized and useful data regarding the system of organ procurement and transplantation and make it widely available to independent investigators and scientific reviewers in a timely manner. The Department of Health and Human Services should provide an independent, objective assessment of the quality and effectiveness of the data that are collected and how they are analyzed and disseminated by the OPTN."

###